Poster #P488

A European Study of the Effectiveness of Risk Minimisation Measures for Fenfluramine Oral Solution in Dravet Syndrome and Lennox-Gastaut Syndrome

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QUESTION

- What is the European physician awareness, knowledge, and self-reported compliance regarding fenfluramine (FINTEPLA®) risk minimisation measures when prescribing fenfluramine for patients with Dravet syndrome (DS) and Lennox-Gastaut syndrome (LGS)?

INVESTIGATION

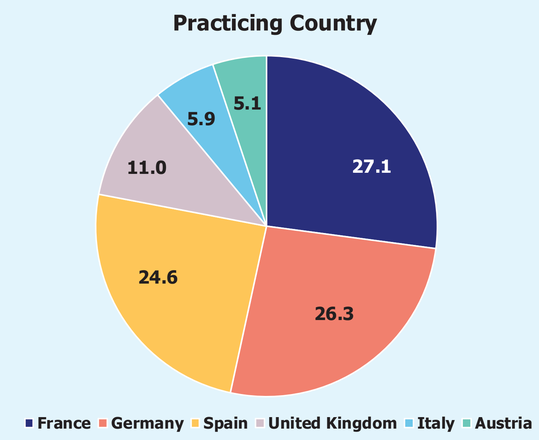
- DS and LGS are rare, developmental and epileptic encephalopathies that are characterised by high seizure burden and severe cognitive, behavioural, and motor impairments[1,2]
- Fenfluramine oral solution is approved as add-on therapy for seizures associated with DS and LGS in patients ≥2 years in the EU[3] and UK[4]; fenfluramine is also approved in other countries/regions, indications may vary
- Due to cardiovascular findings originally identified when the fenfluramine oral tablet was used at high doses (60–120 mg/day) as an anorectic agent, echocardiogram (ECHO) monitoring is required regularly in patients on fenfluramine to detect possible early signs of valvular heart disease (VHD) or pulmonary arterial hypertension (PAH)
- In Europe and UK, ECHO monitoring must be conducted at baseline (prior to fenfluramine start) and then every 6 months for the first 2 years, annually thereafter; an ECHO must also be conducted 3–6 months after discontinuing fenfluramine[3,4]
- Risk minimisation measures also include the use of fenfluramine educational materials for prescribers, patients and caregivers that describe cardiovascular risks and requirement for monitoring, as well as information to prevent off-label use of fenfluramine for weight management
  - These measures are also emphasized for physicians via the Controlled Access Program (CAP) through which fenfluramine is prescribed and dispensed to ensure its safe use
- Here, we present the survey data on awareness, knowledge, and self-reported physician compliance regarding fenfluramine safety in Europe

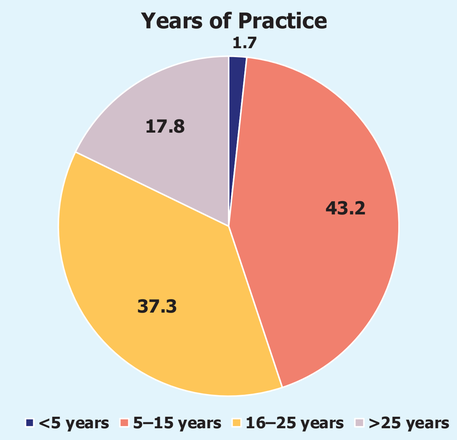
RESULTS

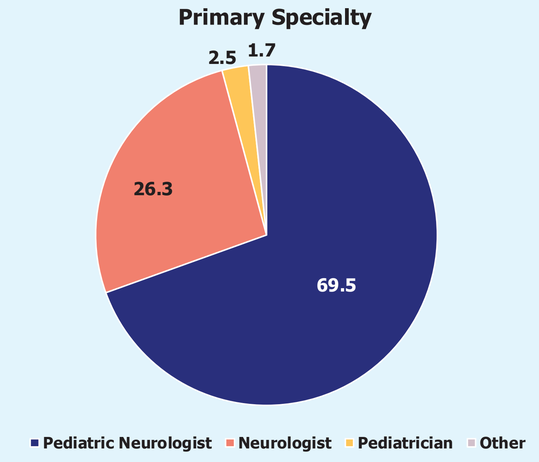
Overview of Physician Participation

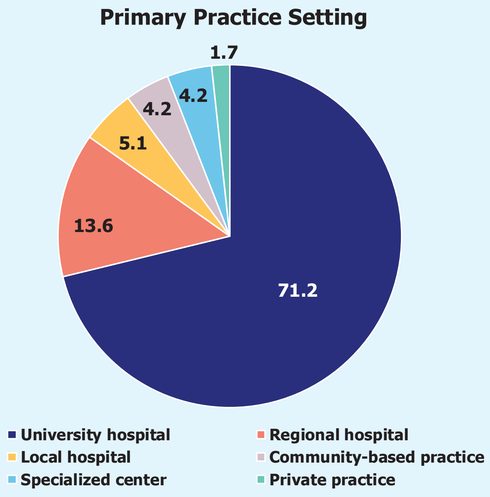
705 invitation letters were distributed → 157 (23.1%) physicians accessed the survey → 119 (75.8%) were eligible to participate → 118 eligible physicians (99.2%) completed the survey

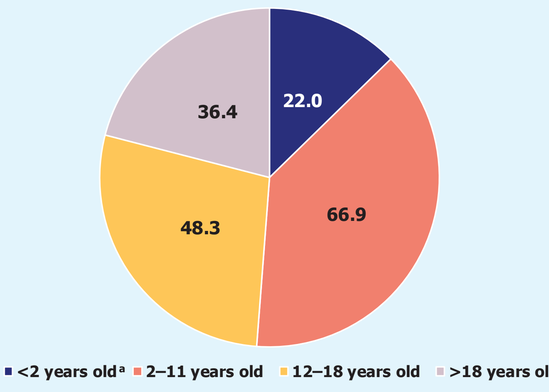
Characteristics of Physicians Who Completed the Survey

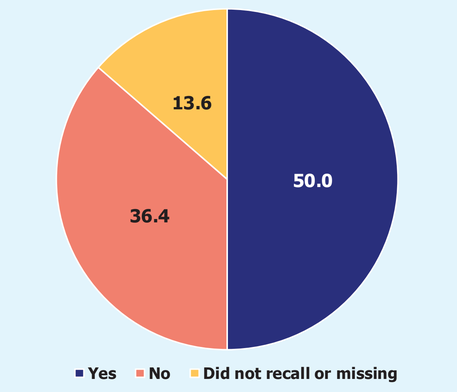








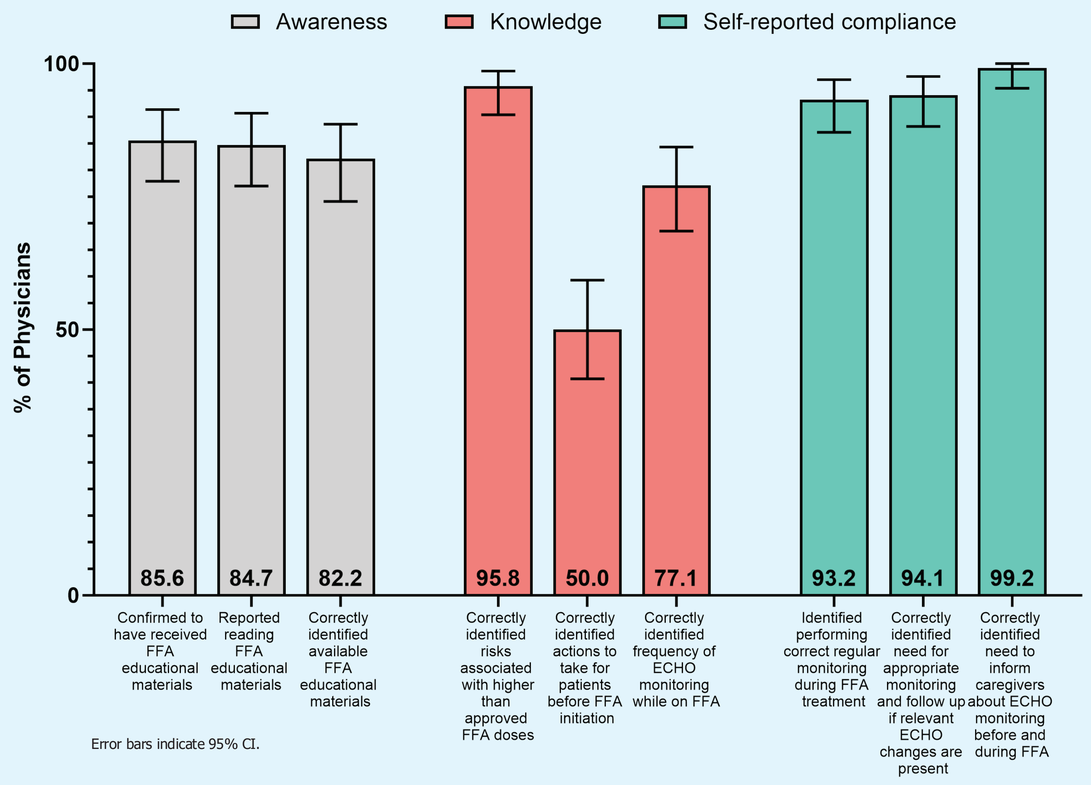




[a]Use in patients <2 years old is off label.
[b]The FFA safety registry (Study EP0218, EUPAS105358) is a requirement from the Pharmacovigilance Risk Assessment Committee to assess long-term cardiac safety of FFA as prescribed in routine clinical practice in select European countries.

Over 50% of prescribers *first* prescribed fenfluramine ≥6 months ago and had *last* prescribed fenfluramine ≤5 months ago.

Correct Responses to Survey Questions Supporting the Primary Objective Domains of Awareness, Knowledge, and Self-reported Compliance (N=118)



Error bars indicate 95% CI.

24/118 (20.3%) of physicians (95% CI: 13.5 to 28.7) provided correct responses to ALL 9 questions.

Proportion of Physicians Who Correctly Responded to All Three Questions Per Primary Objective Domain

Correct Responses to:	N=118
All 3 Awareness Questions, n (%)	**85 (72.0) (95% CI: 63.0 to 79.9)**
All 3 Knowledge Questions, n (%)	**43 (36.4) (95% CI: 27.8 to 45.8)**
All 3 Compliance Questions, n (%)	**103 (87.3) (95% CI: 79.9 to 92.7)**

Mean % of correct responses to *any* questions supporting the primary objectives per survey participant was 84.7% (95% CI: 82.5 to 86.8).

Physician-reported Distribution of Educational Materials and Off-Label Prescribing of FFA for Weight Management

- Physicians reported distribution of educational materials and latest version of the package leaflet to patients/caregivers → **60/118 (50.8%) [95% CI: 41.5 to 60.2]**
- Physicians confirmed they would not prescribe FFA for weight management → **113/118 (95.8%) [95% CI: 90.4 to 98.6]**
- Physicians were aware they should inform patients/caregivers about negative benefit-risk of FFA for weight management → **71/118 (60.2%) [95% CI: 50.7 to 69.1]**
- Physicians correctly identified the content included in FFA educational materials → **59/118 (50.0%) [95% CI: 40.7 to 59.3]**

27/118 (22.9%) of physicians (95% CI: 15.7 to 31.5) provided correct responses to ALL 4 questions.
Mean % of correct responses to *any* question per survey respondent was 64.2% (95% CI: 59.3 to 69.1).

Abbreviations: CI, confidence interval; ECHO, echocardiogram; FFA, fenfluramine.

METHODS

- In this non-interventional, cross-sectional postmarketing safety study, a secure electronic survey of closed-ended questions was delivered to physicians routinely prescribing fenfluramine
- Data were collected 12–18 months after educational program implementation in each country
- The primary objective was to assess effectiveness of the fenfluramine educational program in the domains of **awareness**, **knowledge**, and **self-reported physician compliance**
  - Success criterion for effectiveness was defined as ≥80% of physicians providing correct responses to all nine survey questions related to awareness, knowledge, and self-reported compliance
- Additionally, secondary objectives were: to assess the physician-reported distribution of educational materials to patients/caregivers; and to assess the awareness, knowledge, and self-reported physician compliance regarding physician-specific educational material on prevention of fenfluramine off-label use for weight management
- Descriptive statistics were used and 95% confidence intervals were calculated with the Clopper-Pearson method

CONCLUSIONS

- These data indicate a reasonable degree of awareness among European physicians regarding fenfluramine educational materials, including their role in informing patients/caregivers about cardiovascular risks and off label use, despite the effectiveness success criterion not being met
- Physicians indicated being aware of VHD and PAH risks when prescribing fenfluramine and demonstrated high compliance with echocardiogram monitoring
- Physicians demonstrated a high level of awareness about the benefit-risk of prescribing fenfluramine for weight management; thus, the risk of off-label fenfluramine use for weight management can be considered minimised
- Regular and ongoing distribution of educational materials to prescribers, patients, and caregivers is needed to re-emphasize risk minimisation measures

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Disclosures

All authors are employees of UCB with stock ownership.

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